

In Search of Treatment or a Cure for LGMD?

Advocacy groups, research sponsors and community leaders are frequently contacted by LGMD patients and/or family members asking whether they should participate in different “treatments” that are either being offered somewhere that they heard about over the internet, that a friend recommended or that they heard may “possibly” be available in the future...such as a form of stem cell therapy, gene therapy, snake oil treatments, green tomatoes, vitamins, medication, etc.

SO, HOW DOES A PATIENT WHO FEELS DESPERATE AND WILLING TO TRY ANYTHING THAT MAY HELP THEM MAKE INFORMED DECISIONS?

Patients can empower themselves by investigating whether a treatment has been published and proven to be safe and effective. For the health and safety of the LGMD patient, it is extremely important to determine whether a treatment is legitimate!

TO ASSIST YOU IN ASSESSING THE LEGITIMACY OF A PROPOSED TREATMENT FOR LGMD, PLEASE CONSIDER THE FOLLOWING:

5 QUESTION “CHECKLIST” FOR ASSESSING THE LEGITIMACY OF PROPOSED TREATMENTS

- Has the treatment been validated in a formal clinical trial?
 - Currently, NO treatments for LGMDs have yet been formally validated.
- Is this advertised as a “clinical trial”?
 - If so, is it listed on the website: www.clinicaltrials.gov? (ClinicalTrials.gov is a registry and results database of publicly & privately supported clinical studies of human participants conducted around the world.)
- What is the basis of the treatment's hypothetical effect (i.e., how is this going to make the person better)?
 - Is the proposed efficacy supported by data in published papers?
- For which conditions will the proposed treatment apply?
 - Claims that propose things like "treat muscular dystrophies with stem cells" is very vague and should arouse suspicion, because there are many different types of MD plus there are many types and ways of administering stem cells.
 - Treatments that purport to be a cure-all for too many conditions should arouse suspicion.
- What safety precautions are being followed during the treatment?
 - Who is performing the treatment and what medical precautions are being adhered to during the treatment or procedure?
 - Has approval by regulatory agencies (for example the FDA in the U.S.) been granted? In which countries and for what indications?